18 December 2019
EMADOC-628903358-1296

Public summary of opinion on orphan designation

Veledimex for the treatment of glioma

On 21 August 2019, orphan designation EU/3/19/2199 was granted by the European Commission to Ziopharm Oncology Limited, Ireland, for veledimex for the treatment of glioma.

What is glioma?

Glioma is a brain tumour that affects glial cells (cells that surround and support nerve cells). It mainly affects adults aged over 45 years, but it can occur at any age. Patients with glioma can have severe symptoms. The nature of their symptoms depends on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, muscle weakness in one part of the body and changes in personality, mood, mental capacity and concentration. About one-fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a long-term debilitating and life-threatening disease because of the severe damage to the brain, and it is associated with poor long-term survival.

What is the estimated number of patients affected by the condition?

At the time of designation, glioma affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several medicines were authorised for the treatment of glioma in the EU. Treatments for glioma included surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer). Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 518,400,000 (Eurostat 2019).

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The sponsor has provided sufficient information to show that the medicine (in combination with another medicine called 'adenoviral vector serotype 5 encoding the human interleukin-12 p70 transgene under the control of activator ligand veledimex') might be of significant benefit for patients with glioma because early studies showed that patients lived longer compared to those who received the usual treatment for glioma in previous studies. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Veledimex, which the patient takes by mouth, switches on a gene in another medicine injected into the tumour called 'adenoviral vector serotype 5 encoding the human interleukin-12 p70 transgene under the control of activator ligand veledimex'. Once switched on, the gene produces interleukin-12 (IL-12), a protein that activates the immune system (the body's defences). Producing IL-12 in the tumour is expected to cause the immune system to fight the cancer, thereby leading to improvement of symptoms.

What is the stage of development of this medicine?

The effects of veledimex have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with veledimex in patients with glioma were ongoing.

At the time of submission, veledimex was not authorised anywhere in the EU for the treatment of glioma. Orphan designation of veledimex had been granted in the United States for malignant glioma.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 July 2019, recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Veledimex	Treatment of glioma
Bulgarian	Веледимекс	Лечение на глиома
Croatian	Veledimeks	Liječenje glioma
Czech	Veledimex	Léčba gliomů
Danish	Veledimex	Behandling af gliom
Dutch	Veledimex	Behandeling van glioma
Estonian	Veledimeks	Glioomi ravi
Finnish	Veledimeksi	Gliooman hoito
French	Vélédimex	Traitement des gliomes
German	Veledimex	Behandlung von Gliomen
Greek	Veledimex	Θεραπεία του γλοιώματος
Hungarian	Veledimex	Glioma kezelése
Italian	Veledimex	Trattamento del glioma
Latvian	Veledimekss	Gliomas ārstēšana
Lithuanian	Veledimeksas	Gliomos gydymas
Maltese	Veledimex	Kura tal-glioma
Polish	Weledimeks	Leczenie glejaka
Portuguese	Veledimex	Tratamento do glioma
Romanian	Veledimex	Tratamentul gliomului
Slovak	Veledimex	Liečba gliómu
Slovenian	Veledimeks	Zdravljenje glioma
Spanish	Veledimex	Tratamiento del glioma
Swedish	Veledimex	Behandling av gliom
Norwegian	Veledimeks	Behandling av gliom
Icelandic	Veledimex	Meðferð á glíóma

[1] At the time of designation